



Ketoconazole HRA® Granted European Marketing Authorisation as an Orphan Drug for the Treatment of Endogenous Cushing's Syndrome

Paris, FRANCE, December 15, 2014 - HRA Pharma announced that the European Commission has granted, on November 19th, 2014, a marketing authorisation for Ketoconazole HRA® (ketoconazole, 200 mg tablet) as an oral treatment of endogenous Cushing's syndrome in adults and adolescents above the age of 12 years.

The European Commission's decision comes following an accelerated review of the marketing authorisation application made by the European Medicine's Agency Committee for Medicinal Products for Human Use (CHMP) with a positive opinion issued in September 2014.

The Committee for Orphan Medicinal Products (COMP) recommended that the orphan designation status granted in April 2012 be maintained, concluding that Ketoconazole HRA® is of significant benefit to patients affected by this debilitating disease and meets the criteria for designation as an orphan medicinal product.

"At HRA Pharma, we constantly strive to improve the lives of patients suffering from rare diseases by expanding access to our medicines and services worldwide" said Thierry Ondet, Therapeutic Domain Director for Endocrinology at HRA Pharma. "Ketoconazole-containing medicines have been used off-label to treat Cushing's syndrome for more than 30 years. Ketoconazole HRA® is a fully-authorized medicine which provides Cushing's syndrome patients and their healthcare providers with evidence-based information for optimal patients' management. Cushing's syndrome patients and their healthcare providers are the immediate beneficiaries of this status change".

Ketoconazole HRA® is granted a centralised marketing authorisation with unified labelling valid in 31 European countries including the 28 countries of the European Union, as well as member of the European Economic Area. Orphan designation carries a protection of 10 years of market exclusivity. The first launches of Ketoconazole HRA® in Europe are scheduled in the first quarter of 2015. Ketoconazole HRA® is to be prescribed only by physicians specialised in treating Cushing's syndrome as the posology needs to be individualised for each patient. Information will be sent to healthcare professionals to allow them to prescribe the medicine safely and effectively.

ABOUT ENDOGENOUS CUSHING'S SYNDROME

Endogenous Cushing's syndrome is a heterogeneous disorder of diverse origins caused by prolonged exposure to inappropriately high levels of the hormone cortisol. Cushing's syndrome is associated with increased mortality and multisystem morbidity. Endogenous Cushing's syndrome is caused by tumors secreting adrenocorticotropin hormone (ACTH) whether from pituitary or extra-pituitary origin, cortisol, or very rarely corticotropin-releasing hormone. Estimated prevalence of endogenous Cushing's syndrome is 1/26,000 and, in the EU, it has an annual incidence of 1/1,400,000-1/400,000, with a peak incidence at 25-40 years of age (Orphanet). Of these, 65-80 % have pituitary dependent Cushing's syndrome, 20-25% have adrenal Cushing's and 5% have ectopic ACTH production from a non-pituitary tumor. Definitive therapy of endogenous Cushing's syndrome optimally involves tumor resection. Indications for medical therapy include acutely ill patients, preparation for surgery, those for whom surgery is not indicated, or patients who remain hypercortisolemic postoperatively or while awaiting the effectiveness of radiotherapy.

About HRA Pharma

HRA Pharma is a privately-held European pharmaceutical company that designs products, devices and supporting services in niche areas of health and makes them available to doctors and patients worldwide. The company targets therapeutic gaps in the areas of reproductive health and endocrinology, and uses innovative marketing solutions and socially-conscious programs to promote healthy management of drugs and diseases. Headquartered in Paris, France and with subsidiaries across Western Europe, HRA Pharma has built a strong network of R&D, manufacturing, distribution and NGO partners which enables it to satisfy critical patient needs and improve patient health in over 80 countries across the globe. Visit www.hra-pharma.com for more information.

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